PROVIDER POLICIES & PROCEDURES

SPINRAZA™ (NUSINERSEN)

The primary purpose of this document is to assist providers enrolled in the Connecticut Medical Assistance Program (CMAP) with the information needed to support a medical necessity determination for Spinraza™ (nusinersen). By clarifying the information needed for prior authorization of services, HUSKY Health hopes to facilitate timely review of requests so that individuals obtain the medically necessary care they need as quickly as possible.

Spinraza™ (nusinersen) is a prescription medication used to treat Spinal Muscular Atrophy (SMA) in pediatric and adult patients.

CLINICAL GUIDELINE

Coverage guidelines for the use of Spinraza™ (nusinersen) will be made in accordance with the DSS definition of Medical Necessity. The following criteria are guidelines *only*. Coverage guidelines are based on an assessment of the individual and their unique clinical needs. If the guidelines conflict with the definition of Medical Necessity, the definition of Medical Necessity shall prevail. The guidelines are as follows:

Spinraza™ (nusinersen) may be considered medically necessary as an intrathecal injection for SMA Type 1 patients with symptom onset occurring < six (6) months of age if the following criteria is met:

A. Spinraza™ (nusinersen) must be ordered by a neurologist experienced in treating SMA; **AND**
B. The diagnosis of SMA 1 must be made by a neurologist with expertise in diagnosing SMA. For purposes of this guideline, neurologist refers to a board certified neurologist, preferably specializing in pediatric neurology; **AND**
C. Genetic testing has been performed and has confirmed both:
    1. Mutation/deletion in chromosome 5q with:
        a. Homozygous gene deletion; or
        b. Homozygous gene mutation; or
        c. Compound heterozygous mutation; **AND**
    2. At least two copies of SMN2; **AND**
D. The patient is not dependent on either of the following:
    1. Invasive ventilation or tracheostomy
    2. Use of non-invasive ventilation beyond use for naps and night time sleep; **AND**
E. A baseline motor exam has been completed by a physician (neurologist or physiatrist) or physical therapist (specializing in SMA motor exam evaluations and supervised by a neurologist or physiatrist) experienced in treating SMA and utilizing at least one of the following exam instruments to establish baseline motor ability:
    - Hammersmith Infant Neurological Exam (HINE)
    - Hammersmith Functional Motor Scale Expanded (HFMSE)
    - Upper Limb Module (ULM) Test (non-ambulatory)
    - Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND); **AND**

F. Spinraza™ (nusinersen) administration will follow the current FDA Spinraza™ (nusinersen) labeling for dosing protocol.

Spinraza™ (nusinersen) may be considered medically necessary for patients with other SMA types if the following criteria is met:

A. Spinraza™ (nusinersen) must be ordered by a neurologist experienced in treating SMA; **AND**
B. The diagnosis of SMA must be made by a neurologist with expertise in diagnosing SMA. For purposes of this guideline, neurologist refers to a board certified neurologist, preferably specializing in pediatric neurology; **AND**
C. Genetic testing has been performed and has confirmed both:
    1. Mutation/deletion in chromosome 5q with:
        a. Homozygous gene deletion; or
        b. Homozygous gene mutation; or
        c. Compound heterozygous mutation; **AND**
    2. At least two copies of SMN2; **AND**
D. The patient is not dependent on either of the following:
    1. Invasive ventilation or tracheostomy
    2. Use of non-invasive ventilation beyond use for naps and night time sleep; **AND**
E. A baseline motor exam has been completed by a physician (neurologist or physiatrist) or physical therapist (specializing in SMA motor exam evaluations and supervised by a neurologist or physiatrist) experienced in treating SMA and utilizing at least one of the following exam instruments to establish baseline motor ability:
    - Hammersmith Infant Neurological Exam (HINE)
    - Hammersmith Functional Motor Scale Expanded (HFMSE)
    - Upper Limb Module (ULM) Test (non-ambulatory)
    - Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)

    AND
F. Spinraza™ (nusinersen) administration will follow the current FDA Spinraza™ (nusinersen) labeling for dosing protocol; **AND**
G. Documentation from the treating neurologist must demonstrate why the individual should be considered for the administration of Spinraza™ (nusinersen) given the research demonstrating efficacy is limited and equivocal.

Spinraza™ (nusinersen) will be authorized for an additional six (6) months if the individual **meets the above criteria** and is determined to be a "responder" by demonstrating an improved motor ability in repeat motor testing after the 5th dose. To be classified as a "responder", the patient should receive the following score(s) on the motor test used:

- **HINE**:
    - A 2 point increase (or maximum score of 4) in the ability to kick (consistent with improvement by at least 2 milestones); **or**
    - A 1 point increase in motor milestones of head control, rolling, sitting, crawling, standing or walking (consistent with improvement by at least 1 milestone); **and**
    - The individual needs to exhibit improvement or maintenance of previous improvement in more categories of motor milestones than worsening, from pretreatment baseline (net positive improvement).

- **HFMSE**: (Scored as 0, 1 or 2 with maximum of 40). Improvement of at least a 3 point increase in score from pretreatment baseline.
- **ULM**: At least a 2 point increase in score from pretreatment baseline.
- **CHOP INTEND**: At least a 4 point increase in score from pretreatment baseline.

Repeat motor testing should be done at 6 month intervals and must show additional motor improvement or maintenance of the previously demonstrated motor improvement.

The 6 month periodic re-examination must be done by the same examiner as the baseline exam. If this is not possible, the re-examination must be done by another physician (neurologist or a physiatrist) or physical therapist (specializing in SMA motor exam evaluations and supervised by a neurologist or physiatrist) experienced in treating SMA who must use the same exam instrument unless it is determined that the original exam instrument is no longer age appropriate.

Note: If an individual becomes dependent on a mechanical ventilator (defined as requiring mechanical ventilation for > 21 days) while on Spinraza™ (nusinersen), the authorization will no longer be valid.

NOTE: EPSDT Special Provision
Early and Periodic Screening, Diagnosis, and Treatment (EPSDT) is a federal Medicaid requirement that requires the Connecticut Medical Assistance Program (CMAP) to cover services, products, or procedures for Medicaid enrollees under 21 years of age where the service or good is medically necessary health care to correct or ameliorate a defect, physical or mental illness, or a condition identified through a screening examination. The applicable definition of medical necessity is set forth in Conn. Gen. Stat. Section 17b-259b (2011) [ref. CMAP Provider Bulletin PB 2011-36].

PROCEDURE
Prior authorization of Spinraza™ (nusinersen) is required. Coverage determinations will be based upon a review of requested and/or submitted case-specific information.

The following information is needed to review requests for Spinraza™ (nusinersen):
1. Fully completed State of Connecticut, Department of Social Services HUSKY Health Program Spinraza™ (nusinersen) Prior Authorization Request form (to include physician's order and signature);
2. Clinical information supporting the medical necessity of the treatment as outlined above to include genetic testing and baseline motor exam results and medical record documentation from the treating neurologist; and
3. Other information as requested.

Requesting Authorization
Requests for the prior authorization of Spinraza™ (nusinersen) must be submitted by the ordering physician and faxed to the number listed on the request form. Questions regarding this form should be directed to the HUSKY Health Program Utilization Management Department at 1.800.440.5071 (select option for medical authorizations).

Initial Authorization

Initial approval of Spinraza™ (nusinersen) will be for 5 doses ONLY (4 initial loading doses and 1 maintenance dose) to be given in accordance with the current Spinraza™ (nusinersen) FDA label instructions.

Re-authorization
After the initial 5 doses have been administered, the individual must be re-evaluated with the motor exam test used previously to establish baseline motor ability unless it is determined that the original exam instrument is no longer age-appropriate.

This re-examination must be done by the same examiner as the baseline exam. If this is not possible, the re-examination must be done by another physician (neurologist or physiatrist who is experienced in treating SMA) or a physical therapist (specializing in SMA motor exam evaluations and supervised by a neurologist or physiatrist).

EFFECTIVE DATE
This Policy for the prior authorization of Spinraza™ (nusinersen) for individuals covered under the HUSKY Health Program is effective November 1, 2017.

LIMITATIONS
Not Applicable

CODE:

Code	Definition
C9489	Injection, nusinersen, 0.1mg

DEFINITIONS

1. **Current Procedural Terminology (CPT):** The most recent edition of a listing, published by the American Medical Association, of descriptive terms and identifying codes for reporting medical services performed by providers.
2. **HUSKY A**: Connecticut children and their parents or a relative caregiver; and pregnant women may qualify for HUSKY A (also known as Medicaid). Income limits apply.
3. **HUSKY B**: Uninsured children under the age of 19 in higher income households may be eligible for HUSKY B (also known as the Children's Health Insurance Program) depending on their family income level. Family cost-sharing may apply.
4. **HUSKY C**: Connecticut residents who are age 65 or older or residents who are ages 18-64 and who are blind, or have another disability, may qualify for Medicaid coverage under HUSKY C (this includes Medicaid for Employees with Disabilities (MED-Connect), if working). Income and asset limits apply.
5. **HUSKY D**: Connecticut residents who are ages 19-64 without dependent children and who: (1) do not qualify for HUSKY A; (2) do not receive Medicare; and (3) are not pregnant, may qualify for HUSKY D (also known as Medicaid for the Lowest-Income populations).
6. **HUSKY Health Program**: The HUSKY A, HUSKY B, HUSKY C, HUSKY D and HUSKY Limited Benefit programs, collectively.
7. **HUSKY Limited Benefit Program or HUSKY, LBP**: Connecticut's implementation of limited health

insurance coverage under Medicaid for individuals with tuberculosis or for family planning purposes and such coverage is substantially less than the full Medicaid coverage.

8. **Medically Necessary or Medical Necessity**: (as defined in Connecticut General Statutes § 17b-259b) Those health services required to prevent, identify, diagnose, treat, rehabilitate or ameliorate an individual's medical condition, including mental illness, or its effects, in order to attain or maintain the individual's achievable health and independent functioning provided such services are: (1) Consistent with generally-accepted standards of medical practice that are defined as standards that are based on (A) credible scientific evidence published in peer-reviewed medical literature that is generally recognized by the relevant medical community, (B)recommendations of a physician-specialty society, (C) the views of physicians practicing in relevant clinical areas, and (D) any other relevant factors; (2) clinically appropriate in terms of type, frequency, timing, site, extent and duration and considered effective for the individual's illness, injury or disease; (3) not primarily for the convenience of the individual, the individual's health care provider or other health care providers; (4) not more costly than an alternative service or sequence of services at least as likely to produce equivalent therapeutic or diagnostic results as to the diagnosis or treatment of the individual's illness, injury or disease; and (5) based on an assessment of the individual and his or her medical condition.
9. **Prior Authorization**: A process for approving covered services prior to the delivery of the service or initiation of the plan of care based on a determination by CHNCT as to whether the requested service is medically necessary.

ADDITIONAL RESOURCES AND REFERENCES:

1. Spinraza [package insert]. Cambridge, MA: Biogen, Inc., December 2016.
2. Markowitz JA, Singh P, Darras BT. Spinal Muscular Atrophy: A Clinical and Research Update. Pediatric Neurology 46 (2012) 1-12.
3. Sugarman EA, Nagan N, Zhu H, et al. Pan-ethnic carrier screening and prenatal diagnosis for spinal muscular atrophy: clinical laboratory analysis of >72,400 specimens. Eur J Hum Genet 2012; 20:27-32.
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5. Lunn MR, Wang CH. Spinal muscular atrophy. Lancet. 2008 Jun 21;371(9630):2120-33.
6. Ionis Pharmaceuticals, Inc. A Study to Assess the Efficacy and Safety of IONIS-SMN Rx in Infants with Spinal Muscular Atrophy. In: ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). 2000-[cited 2017 Jan 6]. Available from: https://clinicaltrials.gov/show/NCT02193074
7. Ionis Pharmaceuticals, Inc. A Study to Assess the Efficacy and Safety of IONIS-SMN Rx in Patients with Later-onset Spinal Muscular Atrophy. In: ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). 2000-[cited 2017 Jan 6]. Available from: https://clinicaltrials.gov/show/NCT02292537
8. Finkel RS, Chiriboga CA, Vajsar J, et al. Treatment of infantile-onset spinal muscular atrophy with nusinersen: a phase 2, open-label, dose-escalation study. Lancet. 2017 Dec 17; 388(10063):3017-3026.
9. Chiriboga CA, Swoboda KJ, Darras BT, et al. Results from a phase 1 study of nusinersen (ISIS-SMN(Rx)) in children with spinal muscular atrophy. Neurology. 2016 Mar 8;86(10):890-7.
10. Haataja L, Mercuri E, Regev R, et al. Optimality score for the neurologic examination of the infant at 12 and 18 months of age. J Pediatr. 1999 Aug;135(2 Pt 1):153-61.

11. Glanzman AM, O'Hagen JM, McDermott MP, et al. Validation of the Expanded Hammersmith Functional Motor Scale in spinal muscular atrophy type II and III. J Child Neurol. 2011; 26(12):1499-507.
12. O'Hagen JM, Glanzman AM, McDermott MP, et al. An expanded version of the Hammersmith Functional Motor Scale for SMA II and III patients. Neuromuscular disorders: NMD. 2007; 17(9-10):693-7.

PUBLICATION HISTORY

Status	Date	Action Taken
Original publication	October 2017	Approved by DSS on October 23, 2017.